

FIG. 1

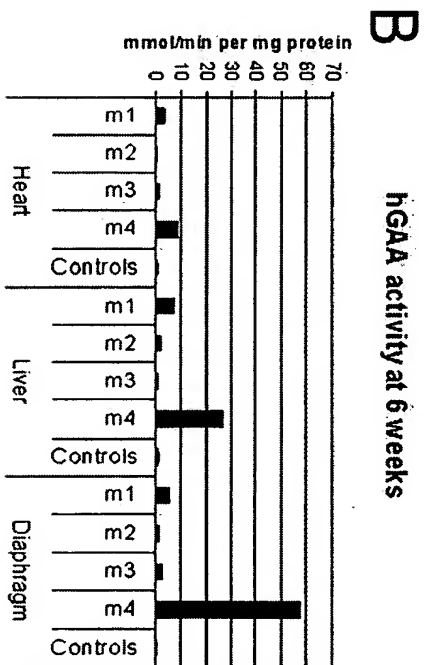
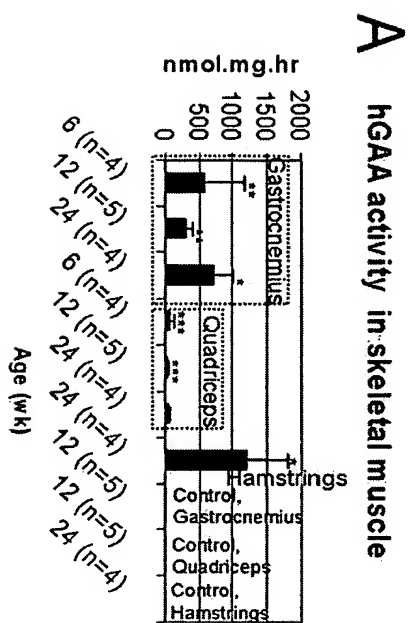
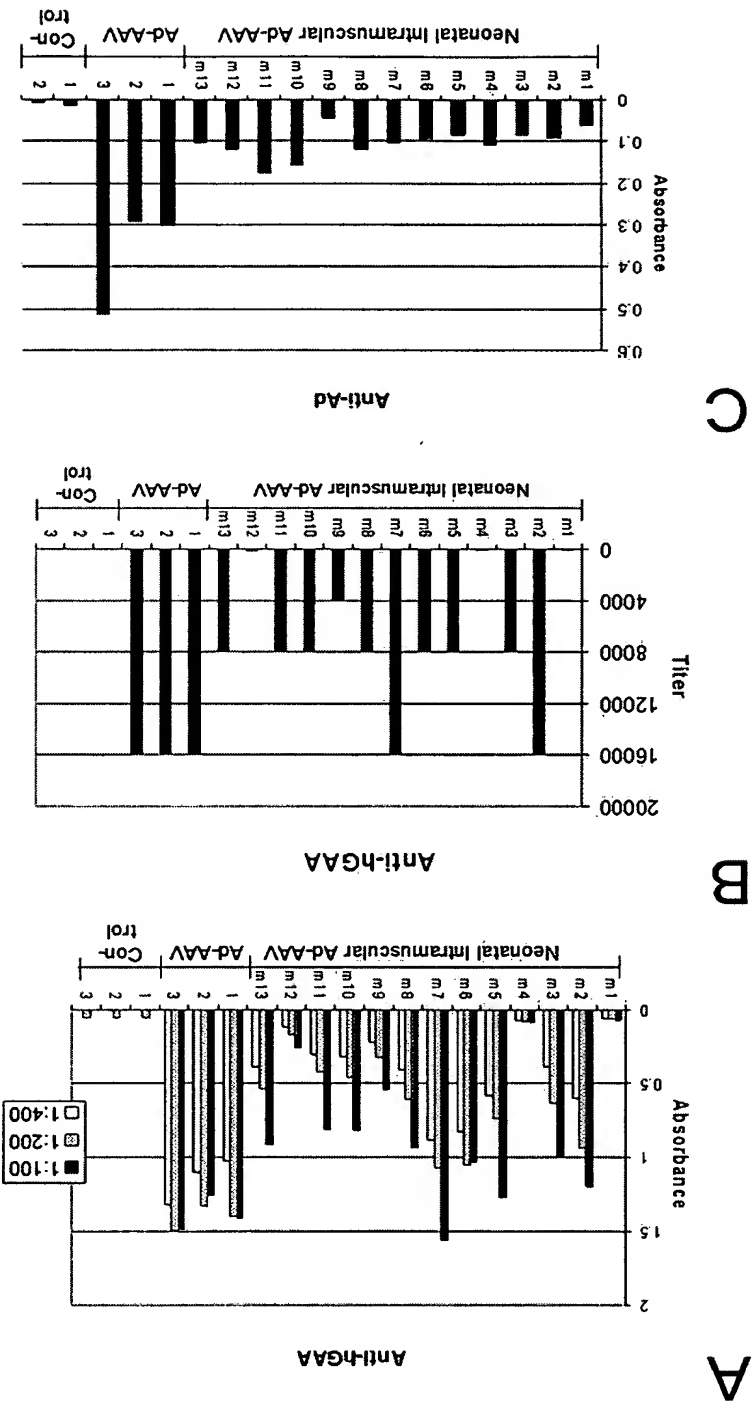


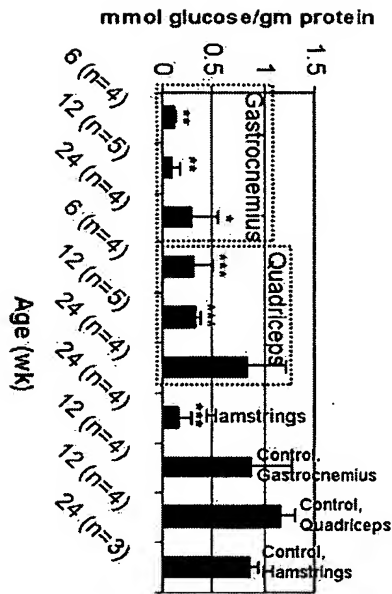
FIG. 2

FIG. 3



A

Glycogen content in skeletal muscle



B

Glycogen content at 6 weeks

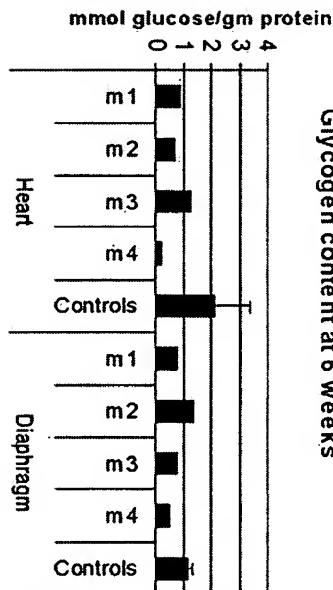


FIG. 4

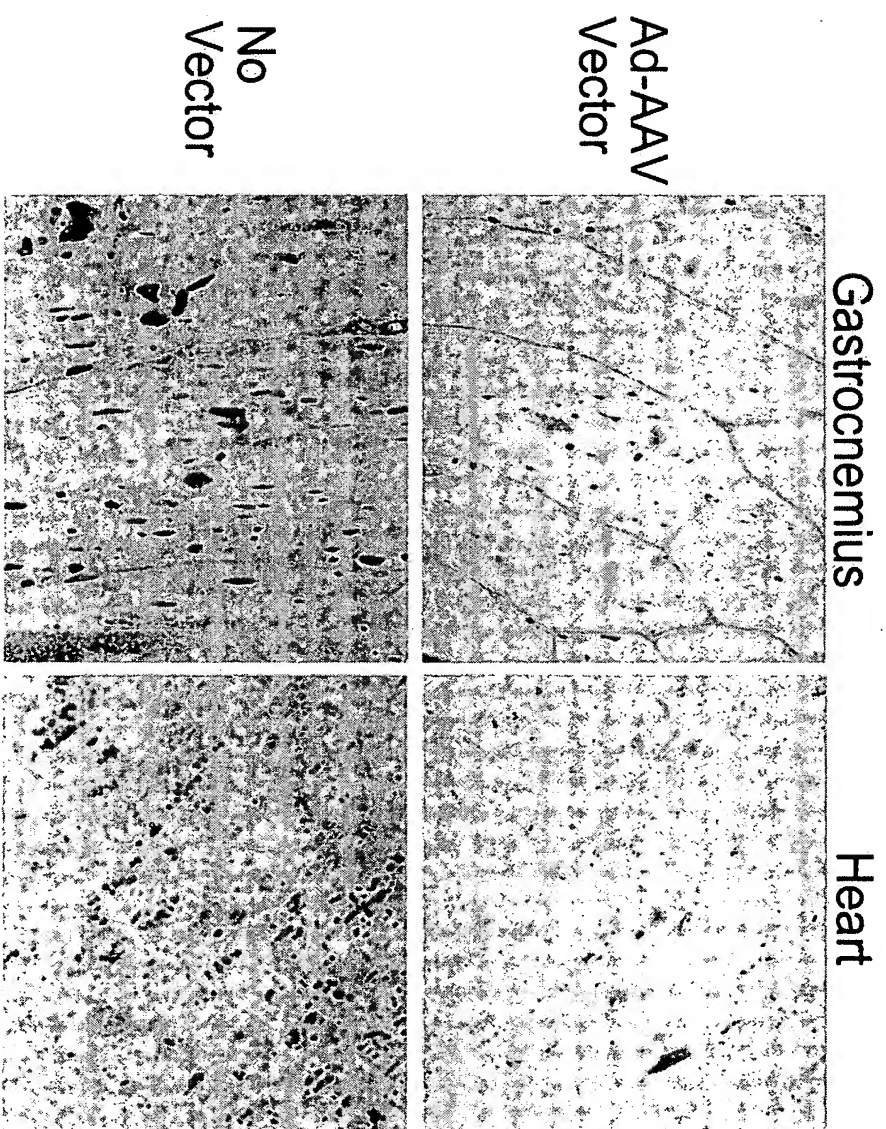


FIG. 5

hGAA with an AAV6 vector in mouse muscle



Reduced glycogen content following AAV6 vector administration

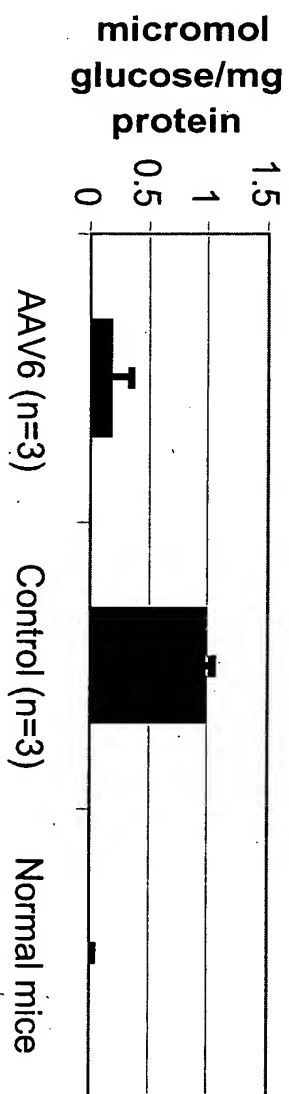


FIG. 6

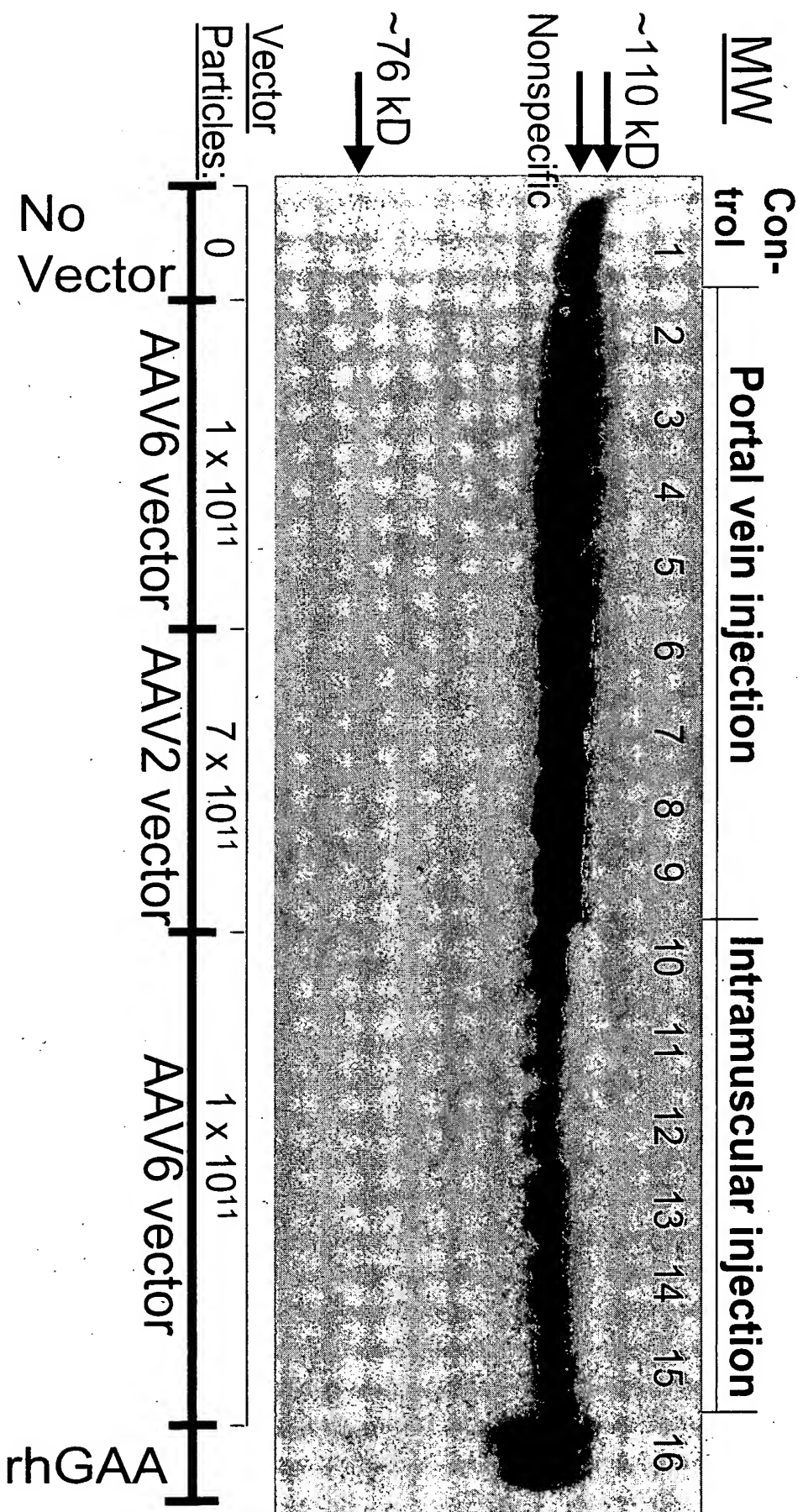


FIG. 7

Figure 8

The full-length human GAA cDNA sequence (3846 bp, Genbank number: NM_000152).

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121 ggccagggcg cgcgtgcgcg gaggtgagcc gggccggggc tgcggggcct cctgagcgc
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241 ttgtcgccgg ggccgcgggt ggaggtcggg gatgaggcag caggtaggac agtgacctg
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361 gaggtcttgg gcctgccgca gctgacgggg aaactgaggc acggagcggg cctgtaggag
421 ctg tccaggc catctcaac catgaggagt aggcacccgc cctgctcca cggctcctg
481 gccgtctgcg cctctgtgtc cttggcaacc gctgcactcc tggggcacat cctactccat
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```


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3721 cggctgccc gagggctgga tgcctgccg tccccagca agcctgggaa ctcaggaaaa
3781 ttacaggac ttgggagatt ctaaacc tta agtgcaatta **tttaataaa** aggggcattt
3841 ggaatc

Figure 9

5' and 3' deleted GAA

1 409 410 g cctgtaggag
421 ctg tccaggc catctccaac **catgggagtg** aggcacccgc cctgctccca ccggctcctg
481 gccgtctgcg cctcgtgtc cttggcaacc gctgcactcc tggggcacat cctactccat
541 gatttctgc tggttccccg agagctgagt ggctcctccc cagtcttga ggagactcac
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2461 cacaacagcc tgctcagct gcccaggag ccgtacagct tcagcgagcc ggcccagcag

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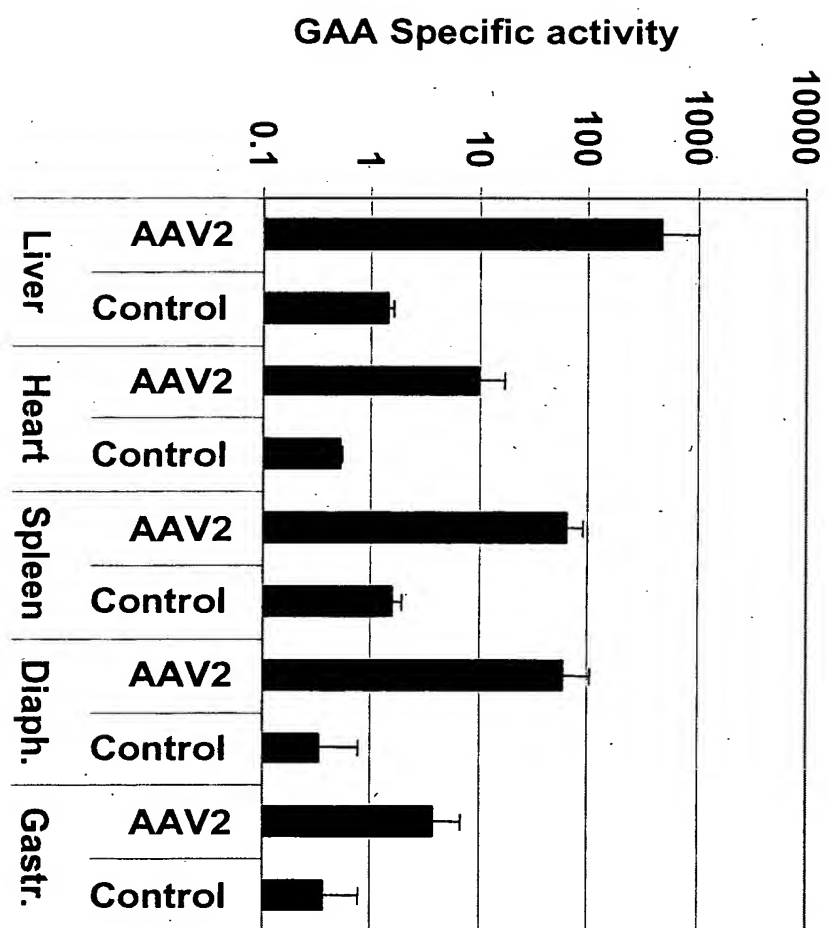


FIG. 10

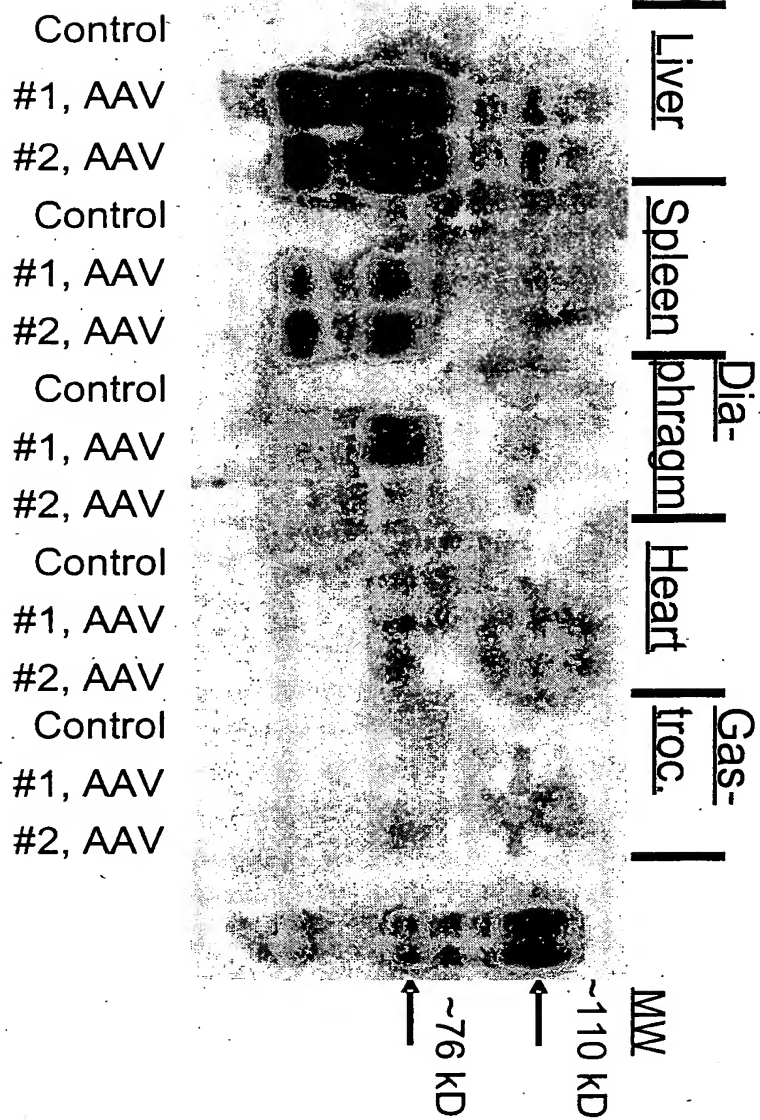


FIG. 11

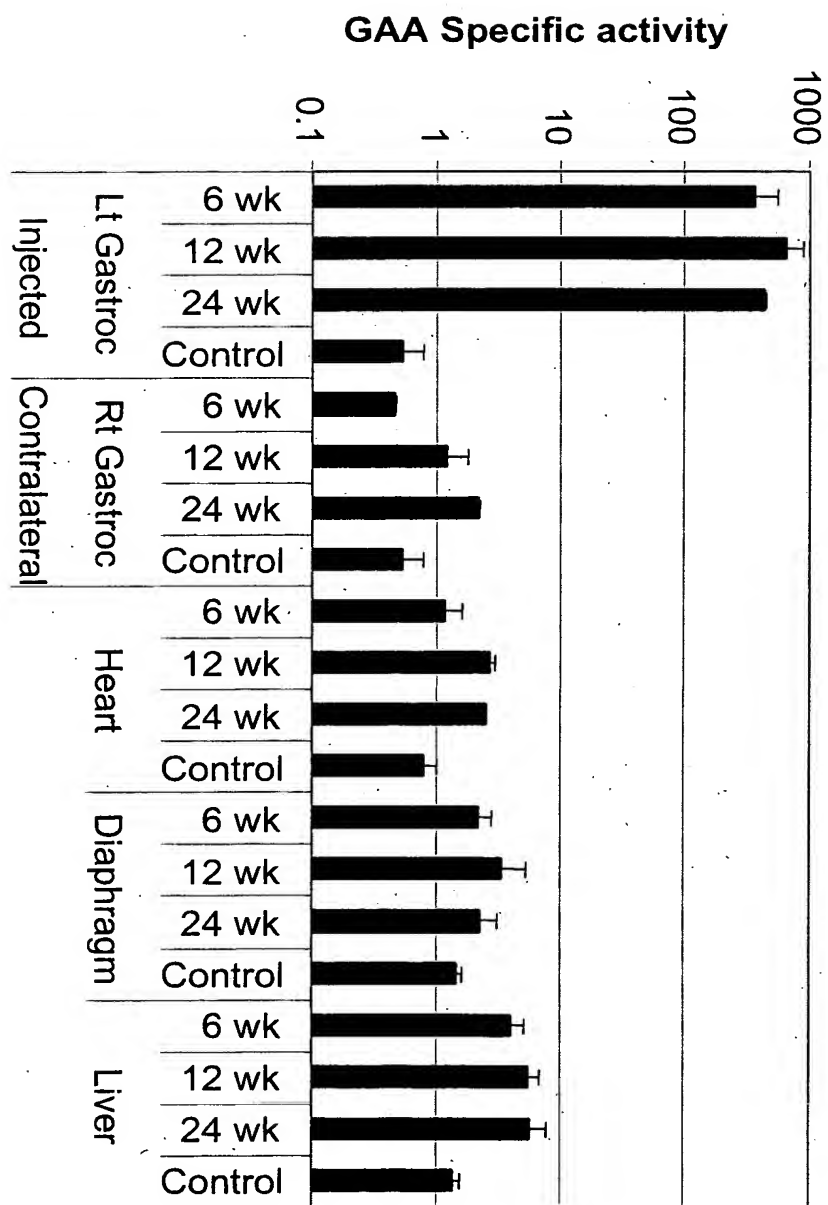


FIG. 12

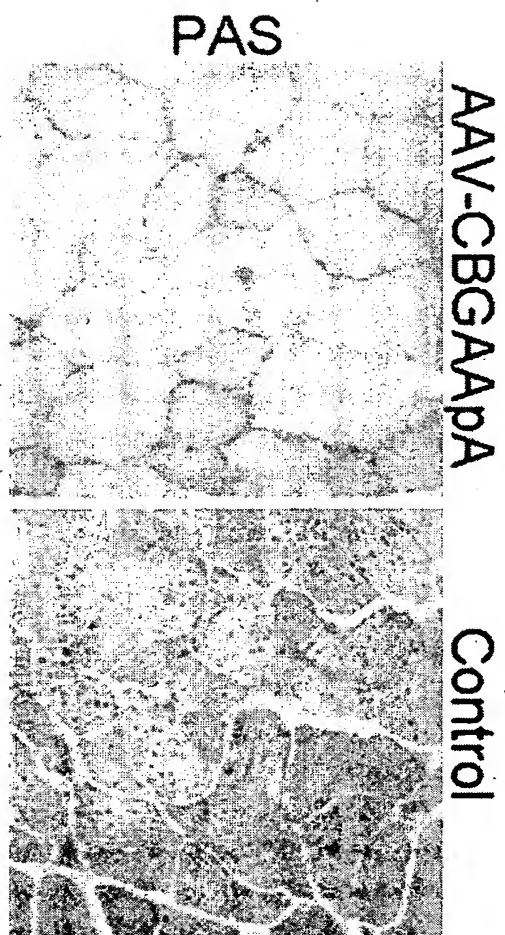


FIG. 13

GAA in transfected 293 cells

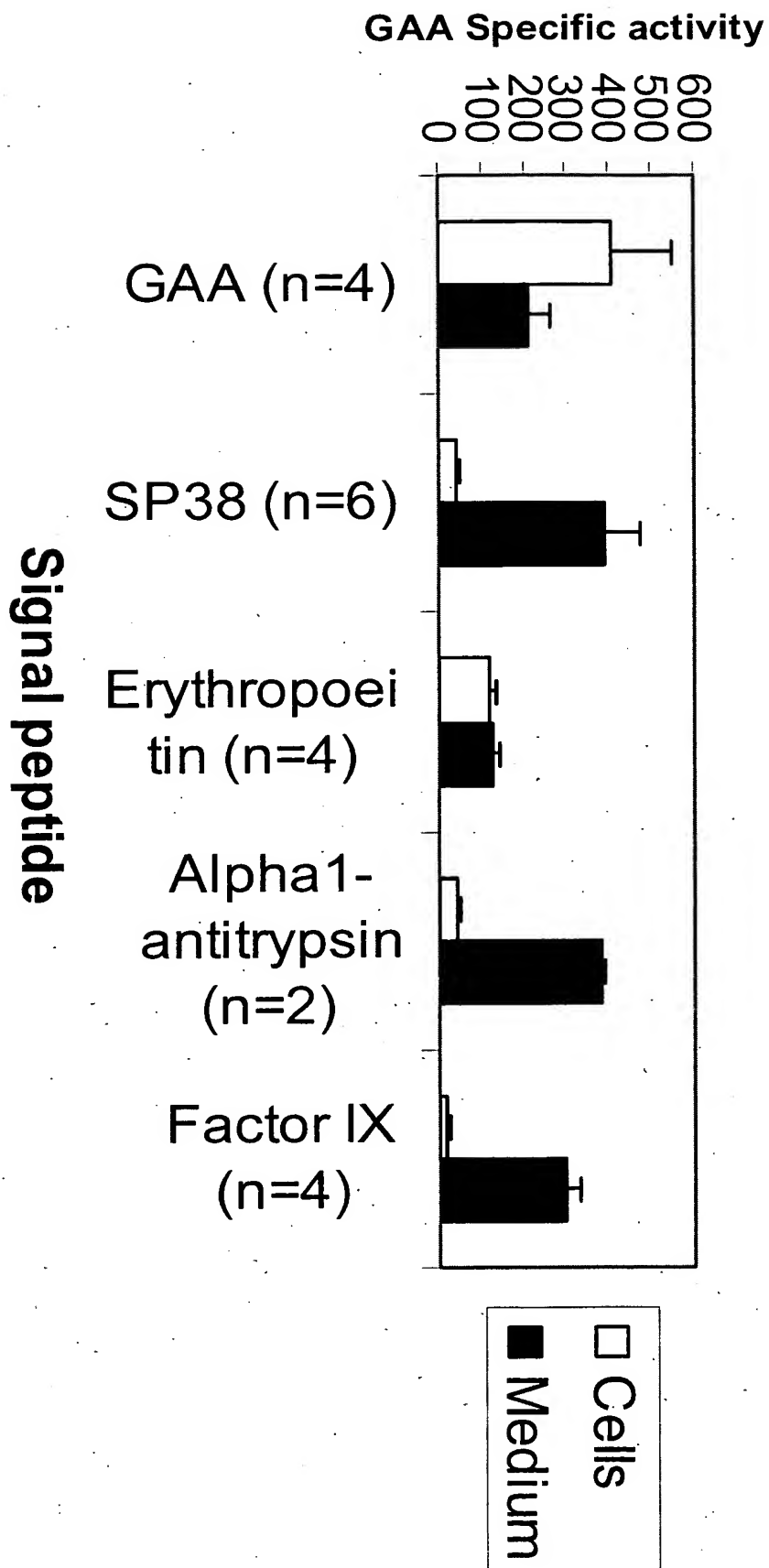


FIG. 14

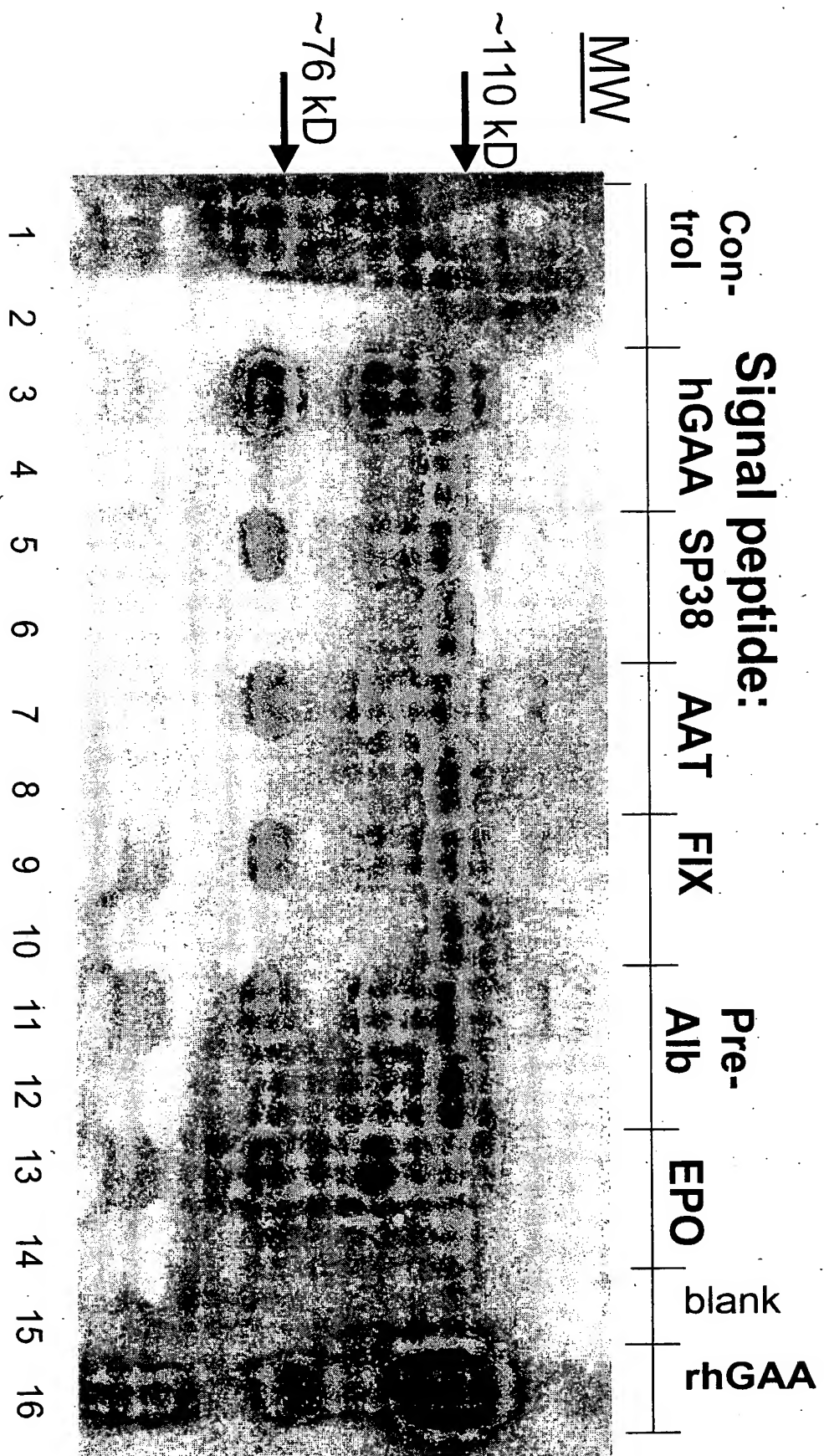


FIG. 15

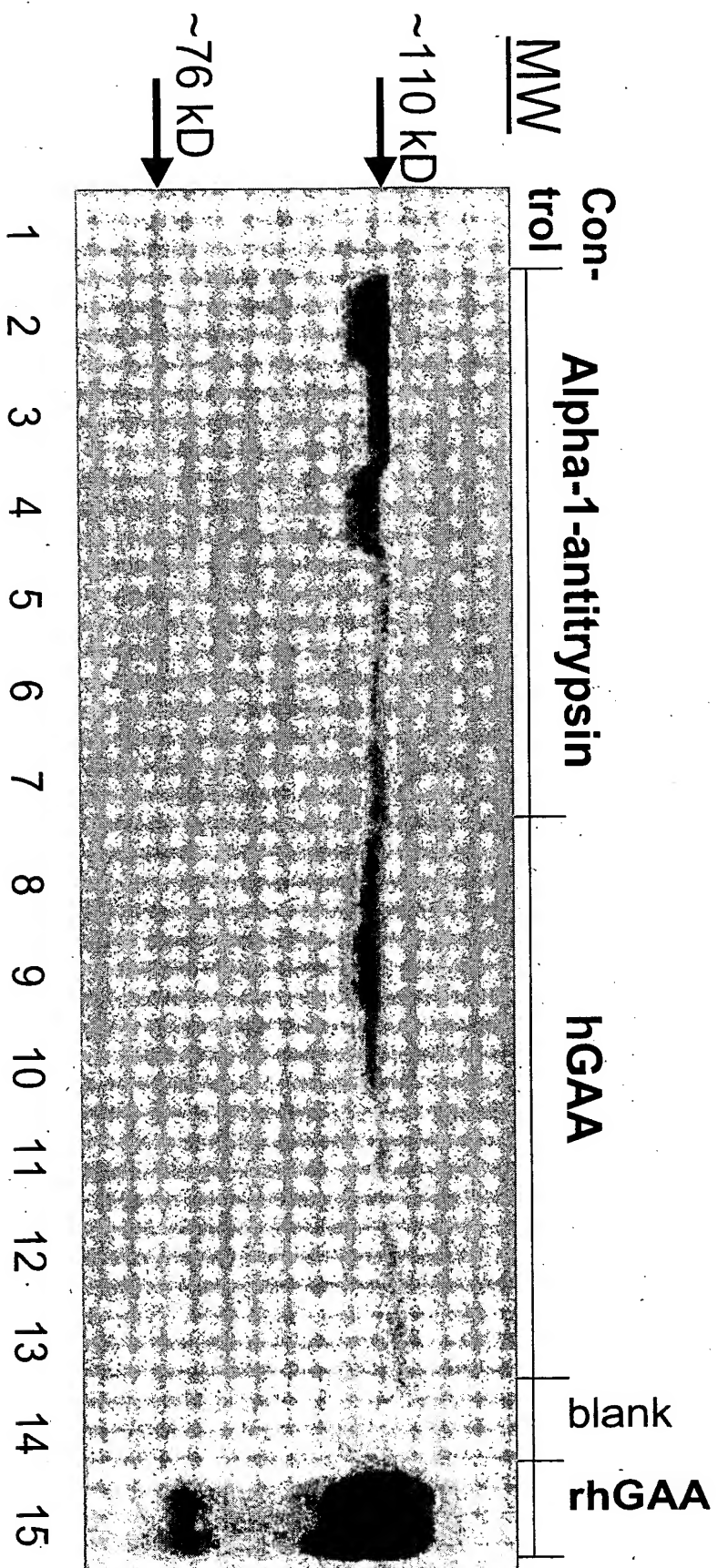
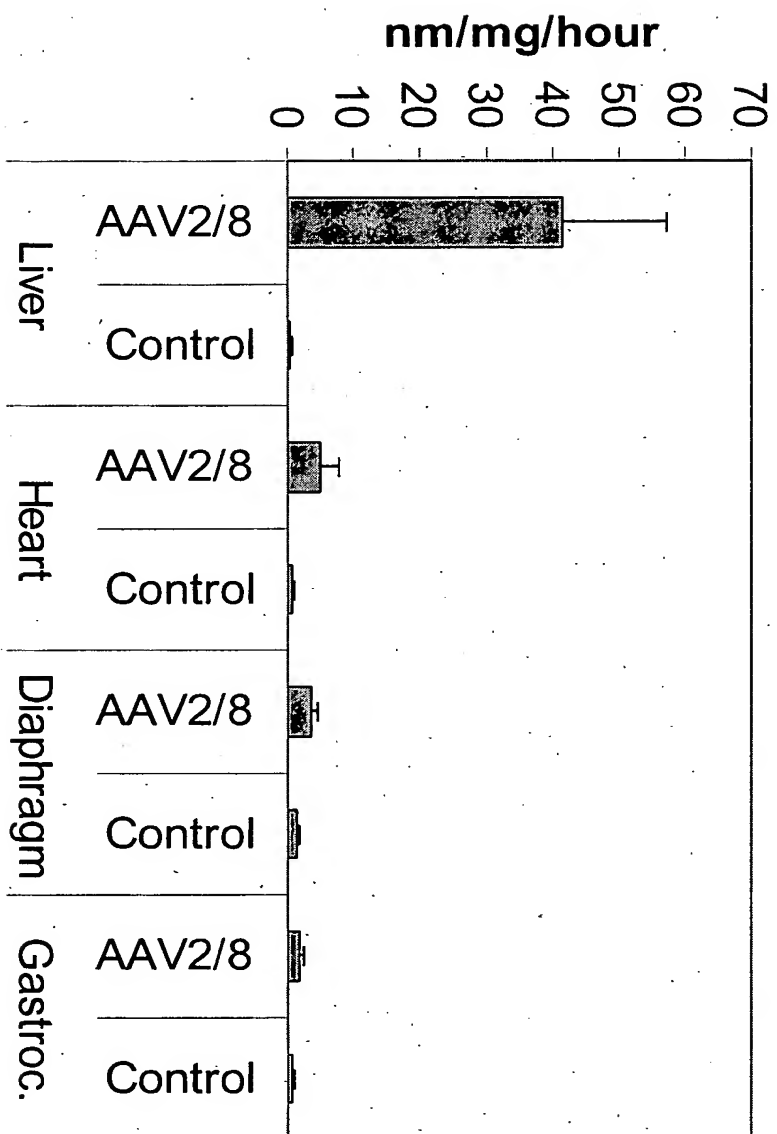


FIG. 16

FIG. 17

**Human GAA with Alpha-1-antitrypsin signal
peptide**



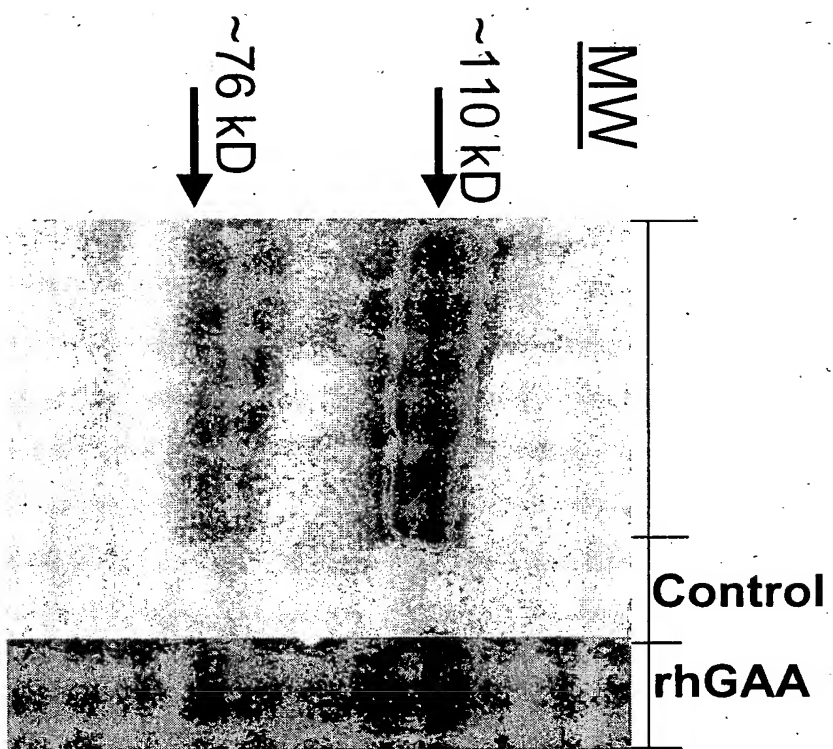


FIG. 18